IN THE UNITED STATES DISTRICT COURT
FOR THE SOUTHERN DISTRICT OF OHIO

The Parent Project for Muscular Dystrophy Research, Inc.,	:	**Case No.**
Plaintiff,	:	**Judge**
v.	:	
Research Institute at Nationwide Children's Hospital, Inc., d/b/a The Abigail Wexner Research Institute,	:	
Defendant.	:	

COMPLAINT

Plaintiff, The Parent Project for Muscular Dystrophy Research, Inc. ("PPMD"), brings this complaint against the Research Institute at Nationwide Children's Hospital ("RINCH"), and states as follows:

Nature of the Action

1. This is an action for breach of contract, indemnification, declaratory judgment, and mandatory injunctive relief arising from a funding award that PPMD provided to RINCH for RINCH research and related work.

Parties

2. PPMD is a not-for-profit organization incorporated under the laws of the District of Columbia, where its main office is also located. PPMD was founded in 1994 by President and Chief Executive Officer Patricia Furlong and others whose family members have been affected by Duchenne Muscular Dystrophy ("DMD"). DMD is a degenerative genetic disease that afflicts mostly boys and leads to death. PPMD seeks to end DMD through funding and

supporting potential treatments and cures for DMD. In furtherance of this effort, PPMD seeks to proportionately share in income that a grantee may receive for commercialization of an invention funded in whole or part by PPMD, so that PPMD may use any monies it receives to invest in or otherwise support new potential DMD treatments or cures.

3. RINCH is a not-for-profit organization incorporated under the laws of Ohio and located in Columbus, Ohio. It is a research entity within Nationwide Children's Hospital ("NCH"). RINCH engages in research to seek new methods of diagnosing, treating, and preventing diseases in children, including DMD. RINCH obtains income from third parties for the development and commercialization of inventions discovered by RINCH employees. RINCH does business as the Abigail Wexner Research Institute.

Jurisdiction and Venue

4. This Court has jurisdiction pursuant to 28 U.S.C. § 1332(a)(1) as the parties are of diverse citizenship and the amount in controversy exceeds $75,000 exclusive of interests and costs.

5. Venue is proper in this Court pursuant to 28 U.S.C. § 1391(b)(1) and (2), as the Defendant resides, and the principal events giving rise to this case took place, in this District and Division.

Facts

Duchenne Muscular Dystrophy

6. DMD is a rare genetic disorder that primarily afflicts boys at an early age. It causes progressive muscle degeneration. Currently, persons with DMD on average are expected to live no later than their 30s.

7. DMD is caused by a mutation of the gene within the human body that facilitates production of dystrophin, a protein located in muscles to help the body function. Dystrophin is one of a group of proteins that work together to strengthen skeletal muscles and the heart muscle. The naturally occurring gene within the human body that facilitates production of dystrophin will be referred to in this Complaint as the "dystrophin gene".

8. Mutations in the dystrophin gene causing DMD do not facilitate production of dystrophin or do not facilitate enough production of dystrophin. If the body does not produce enough dystrophin, muscle cells become damaged and weaken. DMD without treatment is an irreversible condition that gets worse over time. As the disease progresses, it affects the muscles in the shoulders, arms, legs, and trunk, and leads to functional difficulties including raising the arms or walking. In later stages, DMD affects the muscles that assist in breathing and that allow the heart to function.

Microdystrophin Gene Therapy

9. Gene therapy offers a way of slowing or reversing DMD. Gene therapy refers to the process whereby a new gene is added to a person's body to correct the gene that does not function properly. To combat DMD, a new gene is introduced into the human body to cause production of sufficient amounts of dystrophin.

10. In gene therapy, a new gene is introduced into the body through another mechanism called a vector. The vector carries the new gene into the cells of the body. One type of vector used to deliver gene therapy for DMD is called an adeno-associated virus.

11. The dystrophin gene is the largest known gene in the human body. Because the dystrophin gene is so large, it cannot fit into an adeno-associated virus vector. Microdystrophin

is shorter than the dystrophin gene and is used for the new gene because it can fit into the adeno-associated virus for delivery into the human body.

12. A third component of gene therapy is the promoter. The promoter helps the new gene express, or propagate, to the proper cells in the human body. In the case of DMD gene therapy, the promoter assists in introducing the new gene into muscle cells.

Background Leading to the PPMD/RINCH Contract

13. In December 2015, Ms. Patricia Furlong, PPMD's Founding President and Chief Executive Officer, and Dr. Jerry Mendell, a RINCH doctor and research investigator, began additional discussions to have PPMD fund gene therapy work that Dr. Mendell had undertaken to treat DMD. PPMD had previously provided funding to RINCH for Dr. Mendell's gene therapy work at least as early as 2013. The additional discussions that commenced in December 2015 related to the use of microdystrophin for treatment of DMD.

14. Discussions between PPMD and RINCH for PPMD to fund Dr. Mendell's work continued during the first half of 2016, including in June 2016, and thereafter. In an initial written funding proposal provided to PPMD in June 2016 titled "Systemic Delivery of AAVrh.74.MHCK7.microdystrophin for DMD", Dr. Mendell and his colleague Dr. Rodino-Klapac, then also a researcher at RINCH, disclosed to PPMD that they wished to initiate a new study using microdystrophin for DMD based on two observations: they wished to pursue a new approach to delivering the microdystrophin gene through an intravenous delivery system rather than to isolated limbs; and, they believed that a new "MHCK" promoter enabled equal or better assistance to skeletal muscles and at the same time provided a far greater positive effect on the heart muscle than the promoter they had been using with the microdystrophin gene therapy.

Doctors Mendell and Rodino-Klapac requested funding from PPMD in the amount of around $2.2 million.

15. On July 7, 2016, Ms. Furlong sent Dr. Mendell a letter committing up to $2.2 million to fund Dr. Mendell's microdystrophin research. The PPMD funding would support a first study of the microdystrophin technology with the MHCK promoter that would be administered intravenously. In her July 7, 2016 letter to Dr. Mendell, Ms. Furlong also explained that the parties would enter into a contract that would govern PPMD's funding.

16. On August 3, 2016, PPMD entered into a RINCH Confidentiality Agreement effective June 1, 2016 relating to the disclosure of information regarding RINCH's gene therapy technology for DMD and related technologies. On August 22, 2016, Dr. Mendell and Dr. Rodino-Klapac provided an amended research and funding proposal to PPMD. The amended proposal included revised details related to the research that would be conducted with PPMD's funding.

The PPMD/RINCH Contract

17. On or about September 6, 2016, RINCH executed a written contract (the "Contract") sent by PPMD to RINCH on or about August 25, 2016, called an Investigator Award, in which PPMD agreed to provide funding to RINCH in the amount of $2.2 million to support this research. The Contract provided that the PPMD funding would be used for the study of systemic delivery of the microdystrophin technology with the MHCK promoter. The Contract is attached at Exhibit 1 to this Complaint.

18. PPMD's funding enabled RINCH to acquire and manufacture the necessary materials for the "cassette", the product that includes the microdystrophin, the MHCK promoter and the adeno-associated virus vector. The product would be studied by RINCH initially in

monkeys.[1] PPMD's funding also enabled study of the product for the first time in humans. PPMD's funding also enabled necessary discussions between RINCH and the United States Food and Drug Administration ("FDA"), the government agency in the United States that regulates the study and commercialization of treatments for disease.

19. In exchange for PPMD's funding, RINCH agreed that for inventions funded by PPMD in whole or part, PPMD would "participate in the income derived from the invention", "in proportion to PPMD's portion of support for the work or research-giving rise to the invention." Exhibit 1 at page 10.

20. An "invention" is defined in the Contract as "any discovery, material, method, process, product, program, software or use, whether or not patented or patentable or copyrighted or copyrightable, that has an application of value such that its use, licensing, lease or sale can generate revenue." *Id*.

21. PPMD's funding enabled RINCH to devise a new product, namely: **"Systemic Delivery of AAVrh.74.MHCK7.microdystrophin for DMD".** *Id*. at page 1 (bold face in original). This product is an invention (the "Invention") under the Contract.

22. On information and belief, the proportion of PPMD's portion of support for the work or research giving rise to the Invention is 7.16%. This is the proportion of RINCH income from the Invention that PPMD is entitled to receive under the Contract.

Events Subsequent to the PPMD/RINCH Contract

23. On January 10, 2017, Sarepta Therapeutics, Inc. ("Sarepta"), a medical research and drug development company, announced its entry into a research and option agreement with

[1] Because monkeys have many genetic characteristics similar to human beings, new technologies often are first tried in monkeys in an effort to determine whether they will be safe and/or effective in human beings.

RINCH's parent entity, NCH, regarding NCH's microdystrophin gene therapy program, headed by Dr. Mendell and Dr. Rodino-Klapac. In its announcement, Sarepta also stated the following:

> Parent Project Muscular Dystrophy (PPMD) has committed 2.2 million dollars to the trial, with support from additional Duchenne foundations and families. Sarepta has committed to the trial through a separate research agreement with Nationwide Children's, and has an exclusive option to license the program. **PPMD's grant provided incentive for Sarepta to help expand and accelerate this opportunity**.

(Bold face added).

24. PPMD disbursed the Contract funding to RINCH between February 2017 and August 2018.

25. The PPMD funding providing for the first in-human study of the Invention was publicly announced as successful by Sarepta on June 19, 2018. In its public announcement, Sarepta again acknowledged that "PPMD committed $2.2 million to the trial, with support from additional Duchenne foundations and families." Sarepta also included in its public announcement a statement from Ms. Furlong acknowledging that PPMD's funding had been provided at "a critical moment in development" of the Invention.

26. The Invention funded in part by PPMD continues to be studied with the hope that it will prove helpful to persons with DMD such that it will be approved by the FDA and other regulatory agencies around the world as a product for commercial use in humans.

27. Sarepta has applied to the FDA for approval to sell the Invention for use in patients with DMD. The FDA has not yet determined whether the Invention is approved for commercial sale. Currently, the Invention is scheduled for FDA decision in mid-2023.

RINCH's Receipt of Income from Sarepta

28. On October 8, 2018, RINCH and Sarepta entered into a license agreement, whereby Sarepta agreed to pay RINCH certain amounts for the Invention for which PPMD provided funding.

29. RINCH already has received income of more than $38 million from Sarepta for the Invention.

30. RINCH may be due to receive many more millions of dollars in income from Sarepta for the Invention, particularly if the FDA approves the Invention for commercial sale.

RINCH's Undervaluation of Amounts Owed to PPMD

31. RINCH has made certain initial payments to PPMD that are substantially less than the proportion of PPMD's funding for the RINCH work and research giving rise to the Invention.

32. To date, RINCH has made three payments to PPMD in relation to the Invention totaling $1,381,661.60.

33. Each such payment by RINCH to PPMD is based on erroneous assumptions made by RINCH in calculating the amounts that RINCH is obligated to pay to PPMD.

34. PPMD has repeatedly protested to RINCH that it has undervalued PPMD's proportionate share of the Invention income received by RINCH.

35. PPMD has been denied payments owed to it by RINCH in the millions of dollars. On information and belief, RINCH is obligated to pay PPMD an additional $1,431,429 thus far. [2]

[2] PPMD sent RINCH a total of $2,200,000. An amount equal to $75,000 was sent by PPMD to RINCH but apparently not received by RINCH. PPMD did not resend the $75,000 to RINCH. RINCH received and deposited

36. Based on RINCH's conduct and statements to date, to the extent RINCH receives additional income payments for the Invention, RINCH will not willingly make additional payments to PPMD in proportion to the funding provided by PPMD.

37. The Contract between PPMD and RINCH also provides that "[t]o the greatest extent permitted by applicable law, Nationwide Children's Hospital hereby agrees to indemnify, defend and hold PPMD, its directors, trustee, officers, employees, agents and consultants harmless in connection with all liability directly or indirectly arising out of the Investigator Award, including all associated costs, damages and expenses, including reasonable attorney's fees." Exhibit 1 at page 9.

38. PPMD has incurred costs, damages and expenses, including reasonable attorney's fees, in connection with liability arising out of the Contract as a result of RINCH's refusals to pay proper amounts owed to PPMD and provide PPMD necessary information on a timely basis to properly determine the correct amounts of money due to PPMD.

Claims for Relief

COUNT I

Breach of Contract

39. PPMD realleges and incorporates by reference the allegations set forth in paragraphs 1-38, above.

40. RINCH entered into the Contract with PPMD. In consideration for PPMD's grant of $2.2 million to RINCH, RINCH and PPMD agreed that PPMD would participate in the

all other payments, *i.e.,* $2,125,000, made by PPMD in satisfaction of PPMD's obligation to pay RINCH $2.2 million under the Contract. PPMD's statement of the amount it has been underpaid is based on the $2,125,000 amount.

income derived from the Invention in proportion to PPMD's portion of support for the work or research giving rise to the Invention.

41. PPMD performed its obligations under the Contract by making payment to RINCH in the amount of $2.125 million for work and research giving rise to the Invention.

42. RINCH has received more than $38 million in income from the Invention. RINCH, however, has failed to meet its obligations to PPMD under the Contract. RINCH has failed to make full payment to PPMD in proportion to PPMD's funding of the Invention as agreed to in the Contract.

43. By failing to make complete and full payment of money due to PPMD, RINCH has breached the Contract.

44. As a direct and proximate result of RINCH's breach of the Contract, PPMD has been damaged in an amount that is at least $1,431,429, plus interest.

COUNT II

Declaratory and Injunctive Relief

45. PPMD realleges and incorporates by reference the allegations set forth in paragraphs 1-44, above.

46. The Contract between PPMD and RINCH provides that PPMD shall participate in the income derived from the Invention in proportion to PPMD's portion of support for the work or research giving rise to the Invention.

47. RINCH has derived income and, on information and belief, will continue to derive income from the Invention for which PPMD provided funding, through further commercialization of the Invention.

48. Upon information and belief, PPMD's proportionate share of funding related to the Invention is 7.16%. Under the terms of the Contract, PPMD is therefore entitled to 7.16% of RINCH income for the Invention. RINCH, however, has failed, and refuses, to provide the correct and full amount to which PPMD is entitled. Further, RINCH's failures, refusals and other statements to PPMD establish that RINCH will not pay PPMD the proportionate share of future RINCH Invention income to which PPMD is contractually entitled.

49. There exists a substantial case or controversy between the Parties involving considerable sums of money for which a declaratory judgment pursuant to 28 U.S.C. §2201 is necessary and appropriate.

50. PPMD is entitled to a declaration from the Court as to its appropriate proportion of RINCH income from the Invention.

51. PPMD is further entitled to a permanent mandatory injunction requiring RINCH to pay in perpetuity to PPMD its proportionate share of income received by RINCH from the Invention.

COUNT III

Indemnification

52. PPMD realleges and incorporates by reference the allegations set forth in paragraphs 1-51, above.

53. The Contract between PPMD and RINCH provides that NCH will pay PPMD for all costs, damages and expenses, including reasonable attorney's fees, that PPMD incurs in connection with liability arising out of the Contract.

54. PPMD has incurred substantial liability, culminating in this Complaint and the amounts that will be necessary to prosecute this case.

55. PPMD is entitled to receive from RINCH all associated, costs, damages, and expenses, including reasonable attorney's fees, in connection with the liability suffered by PPMD as a result of RINCH's failures to pay PPMD the amounts properly owed to it and by RINCH's refusals to properly calculate PPMD's proportionate amount for purposes of receipt of future RINCH Invention income.

Prayer for Relief

WHEREFORE, Plaintiff PPMD respectfully requests that the Court award the following relief:

A. A declaratory judgment setting forth the appropriate proportion of RINCH income from the Invention to which PPMD is entitled;

B. An award of damages plus interest arising from RINCH's breach of the Contract;

C. An order directing RINCH to pay all amounts due and owing PPMD under the Contract;

D. A permanent mandatory injunction requiring RINCH to pay PPMD in perpetuity its proportionate share of income derived by RINCH from the Invention;

E. An award of PPMD's reasonable attorneys' fees and other costs, fees, and expenses as provided by the Contract; and

F. Such other and further relief as is just, fair, and appropriate.

Respectfully submitted,

Date: December 20, 2022

/s/ David P. Shouvlin

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